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**Remarks by Acting Secretary of Health Carmona Regarding Coverage of Recombinant Factor May be Cause for Concern of Donated Blood**

Acting Secretary of Health Richard Carmona, MD, caused concern among individuals in the bleeding disorders community recently by raising questions about the cost effectiveness of recombinant clotting factor products, without referencing the superior safety of these products. The comments were raised in a response to a January communication from the U.S. Department of Health and Human Services (DHHS) Advisory Committee on Blood Safety and Availability (BSA) recommending the updating of outdated language in the Medicare manual. The language in question could have been used by states to limit reimbursements for recombinant factor. In his response, Acting Secretary Carmona did not address the specific recommendations on language. He chose instead to cite the lack of medical literature demonstrating the enhanced efficacy of recombinant products, and called upon the Centers for Medicare and Medicaid Services (CMS) to conduct a cost-effectiveness analysis of recombinant products.

The BSA is on record as supporting access to recombinant products. In April of 1998, the committee recommended that "every effort should be made to make recombinant clotting factors available to all who would benefit from them, and all barriers to conversion from human plasma-derived concentrates to recombinant clotting factors should be removed." BSA Committee Chair Dr. Mark Brecher has scheduled a meeting with the DHHS at the end of May to convey the serious concerns the committee and many in the bleeding disorders community have about the Acting Secretary's remarks. The meeting will be an opportunity for Dr. Brecher to stress the superior safety of recombinant products based on both known and theoretical risks.

NHF similarly supports access to recombinant products for all persons with a bleeding disorder, and NHF's Medical and Scientific Advisory Council cited the BSA guidance in its own recommendation on the subject (MASAC #106, November 12, 2000).

NHF is carefully monitoring developments related to Acting Secretary Carmona's comments and will convey information to the community as it becomes available. NHF volunteers who serve on the BSA committee and have played a leading role in this effort include Keith Hoots, M.D., Paul Haas, Mark Skinner, and Dana Kuhn. Following Dr. Brecher's meeting, NHF leadership will assess the results and take whatever further action is necessary to protect the community's access to the safest products available. BSA committee recommendations and meeting transcripts are available at



<http://www.hhs.gov/bloodsafety/>.  
NHF MASAC recommendations  
are available at <http://www.hemophilia.org/programs/masac/masac.htm>

**ADVOCACY UPDATE:**

**NHF Broadening Efforts to Include State-based Advocacy**

This year, NHF is broadening its advocacy campaign to include efforts to address needs at the state government level. Financial pressures on the state Medicaid programs have forced individuals in many states to either settle for less than optimal healthcare or fight locally for access to their choice of drugs and medical provider. More than 30% of people with hemophilia are dependent on government subsidized insurance, and it is likely that, as Medicare and Medicaid roll back their reimbursement levels and limit access, private insurers will follow suit. This reality has provided the impetus for NHF to support advocacy at the state level so that all people with bleeding disorders will have equal access to optimal healthcare, no matter what state they live in and no matter what insurance program they participate in.

Last March, at the annual Washington Days program, NHF presented a workshop that included discussions on this important

issue. An additional seminar on the subject will be offered during the annual New York Leadership Weekend in June. A newly formed State Advocacy Task Force, composed of NHF leadership and representatives of chapter leadership, will also begin meeting to address how NHF can best serve chapters in the local advocacy arena. The Task Force will make its recommendations to the NHF Board of Directors in June. Look for update in upcoming editions of eNotes.

**Project Red Flag Briefing Held in Washington DC**

On May 6th, 2003, NHF's Project Red Flag:Real talk about women's bleeding disorders held its first legislative briefing and tea in Washington, DC. Deputy Assistant Secretary of Health and NHF Task Force member Dr. Wanda Jones moderated the program. The 36 legislators and staff in attendance heard presentations by task force member Dr. Andra James of Duke University, Sally Crudder of the Centers for Disease Control and Prevention, and Irene Vlaskamp, physical therapist and woman with von Willebrand disease. Dr. James reviewed the clinical profile of VWD, including identification and treatment. Sally Crudder described the incidence in the general population and in cases where menorrhagia (heavy periods) is presented. Irene Vlaskamp spoke of her 19-year journey to diagnosis and proper care.

Also participating in the briefing were Hemophilia Association of the Capitol Area President Susan Yamamoto, Executive Director, Sandi Qualley, and Melissa Hurtt, a member of the Hemophilia Foundation of Maryland.

**HHS To Award \$80 Million To States To Offset Costs of Insurance For Residents Too Sick For Conventional Coverage**

HHS Secretary Tommy G. Thompson recently announced the availability of \$80 million in grants for states that provide health insurance to residents who can not get conventional health coverage because they are too sick. The grants would be used by states to offset losses they may incur operating high-risk pools, which are typically state-created non-profit association that offers health coverage to individuals with serious medical conditions. Enrollment in these pools is growing, with more than 153,000 individuals enrolled in state pools.

The funding will be awarded over two years, as authorized in the

Trade Adjustment Assistance Reform Act of 2002. To be eligible, a state must have a "qualified" high-risk pool that meets the criteria specified in the Trade Act and must follow such rules as capping premiums at no higher than 150% of the standard charge in the state. States may be eligible for a grant that matches up to 50% of the losses incurred in the operation of the risk pools. Funds will be distributed based on the number of uninsured individuals in each state. HHS' Center for Medicare & Medicaid Services (CMS) will administer the program. To date, 22 states have high-risk pools that meet the "qualified" criteria. In November, HHS also announced the availability of grants of up to \$1 million each for states to use as seed money to establish high-risk pools. A total of \$20 million is available under that program.

More information about risk pools is available at [www.cms.hhs.gov/riskpool](http://www.cms.hhs.gov/riskpool).

Source: HHS Press Release

### **Tests to Screen Blood for West Nile Should be Available by Summer**

Recent statements by the blood bank industry and others indicate that tests capable of detecting West Nile virus in donated blood should be in place in time for mosquito season this summer. The Food and Drug Administration, (FDA), with encouragement from NHF and other groups with an interest in the safety of the blood supply, called for the development of screening tests after it was discovered last year that West Nile virus could be transmitted via blood. Previously, it had been believed that the disease was only transmitted by mosquitoes, but at least 21 of the more than 4,000 people who were infected with the virus appear to have contracted it via blood transfusions or organ transplants.

Chiron in Emeryville, Calif., and Roche Biomedical in Burlington, N.C., are developing two separate blood-screening tests. The tests detect genetic material of the virus using a technique called nucleic acid amplification testing or NAT. Similar tests are already in use to screen for HIV and hepatitis C in the blood supply. Both companies expect to have their tests ready soon. However, the tests must receive FDA approval before they can be distributed. An FDA spokesperson said the agency "will work with (the test manufacturers) closely to try to expedite any review or approval," and that even if the tests do not have full FDA approval, the agency might elect to grant them special consideration and allow them to be used under an investigational protocol.

Source: United Press International

### **Antiretroviral Therapy Prevents Liver Damage in Patients Coinfected with HIV and HCV**

Scientists in Spain investigating "the factors associated with liver fibrosis in human immunodeficiency virus and hepatitis C virus (HIV/HCV) co-infected patients" discovered that antiretroviral treatment can prevent liver damage in these individuals. The research, performed by C. Tural and colleagues at Hospital Universitari Germans Trias i Pujol, consisted of an observational, single-centered, cross-sectional study of 180 HIV/HCV co-infected patients who underwent liver biopsy between May 1998 and November 2001.

Source: Hepatitis Weekly

### **Collaborative Effort between Industry and Academia Yields Results that May Lead to New HIV Treatment**

In a successful partnership between industry and academia, researchers from the University of Maryland, Baltimore County (UMBC), and Achillion Pharmaceuticals have announced the discovery of a new target on the HIV molecule that could potentially lead to a new class of antiviral drugs to fight the virus that causes AIDS.

The study, headed by Dr. Michael



Summers, UMBC professor of chemistry/biochemistry and Howard Hughes Medical Institute (HHMI) Investigator, is featured on the cover of the April 11, 2003, *Journal of Molecular Biology*.

"The greatest challenge in treating HIV today is drug resistance brought on when the virus mutates and renders existing drugs ineffective at stopping viral replication," said Summers. "Our research has led to the identification of a new class of compounds that inhibit a novel target in HIV."

Summers and his team identified the target at the UMBC HHMI Lab and discovered a number of compounds that bound to a specific area of the capsid protein thought to play a key role in the assembly process necessary for HIV to mature to its infectious form. The identification process leveraged the nuclear magnetic resonance (NMR) technology resources and target validation expertise at Summers' HHMI laboratory. Once the new capsid assembly target and compounds inhibiting the target were identified using computer models, researchers from Achillion tested the compounds in a number of biochemical assays, as well as in human cells infected with live HIV. These experiments demonstrated that the anti-HIV activity of inhibitors was indeed due to disruption of the HIV-1 capsid protein. Three patents have been filed based on the findings reported in the publication.

Source: *Anti-Infectives Week*

### **Bayer Biological Products Receives Patent for Western Blot Assay, Allowing Quicker, Less Expensive Confirmation of Removal of Disease-Causing Prions During Manufacturing**

Bayer HealthCare LLC, Biological Products Division (BP), announced recently that it received a Notice of Allowance from the United States Patent Office for a patent covering the use of the Western Blot Assay to confirm removal of pathogenic prion proteins during the manufacture of therapeutic proteins. This test is the first of its kind in the biological products industry.

The patent covers a process Bayer BP uses for a quicker and less expensive assessment of prion protein removal during the manufacture of human plasma-derived products. The pathogenic form of prion proteins has been associated with fatal diseases, including Creutzfeldt-Jakob disease (CJD). To date, no clinical evidence suggests the transmission of human transmissible spongiform encephalopathies (TSEs), such as CJD, by blood or blood plasma-derived products. Manufacturers are working toward eliminating this theoretical risk for transmission. NHF has encouraged this process to ensure all products used by people with bleeding disorders are as safe as possible.

Source: *Business Wire*

### **FDA Approval Granted for Hepatitis C Viral Load Assay**

Bayer HealthCare LLC announced recently that it has received pre-market approval after expedited review from the U.S. Food and Drug Administration (FDA) for its Versant HCV RNA 3.0 Assay (bDNA), a predictive test that directly measures hepatitis C virus RNA levels in serum or plasma.

The Versant HCV viral load assay is the first and only FDA-approved quantitative test to measure HCV viral load levels, and will aid physicians by guiding therapeutic decisions early in treatment. The level of viral load, or HCV RNA, in a patient's blood can identify, early in treatment, patients who may not respond to further therapy. Utilizing an accurate HCV RNA quantitative assay such as the Versant HCV viral load assay can help clinicians decide if therapy should be discontinued, thereby avoiding the unnecessary side effects of prolonged treatment.



FDA granted Bayer an expedited review of its premarket approval application on July 26, 2002. The FDA grants such reviews for products that provide treatment or diagnosis of life-threatening or irretrievably debilitating diseases or conditions and for which no approved alternative exists.

Source: Medical Letter on the CDC & FDA

## NHF NEWS

### **National Hemophilia Foundation Names Ten Institutions as Approved Training Centers for NHF/Baxter Clinical Fellowship Program**

#### **First Fellows to be Announced in June**

NHF announced that ten institutions have received approval as training centers for its Clinical Fellowship Program. The program, which is funded through the generous support of Baxter Healthcare Corporation, is intended to provide encouragement and training for individuals interested in caring for people with bleeding and clotting disorders, and to prepare candidates for academic careers.

The ten institutions and their directors are The Children's Hospital of Philadelphia, Catherine Manno, MD; the University of Texas/Health Science Center, Houston, W. Keith Hoots, MD; The Medical College of Wisconsin, Milwaukee, Joan Gill, MD; Emory University School of Medicine, Atlanta, Thomas Abshire, MD; the University of Iowa, Iowa City, Steven Lentz, MD, PhD; the University of Minnesota, Minneapolis, Nigel S. Key, MD; New York Presbyterian Hospital, New York, Donna DiMichele, MD; Harvard Medical School, Cambridge, MA, Peter Marks, MD, PhD; The University of North Carolina at Chapel Hill, Gilbert C. White II, MD; and the University of Michigan Department of Pediatrics, Ann Arbor, Steven Pipe, MD.

The institutions were obliged to submit detailed applications that outlined their capacity to fulfill the training requirements of the program. Approval at this stage does not guarantee eventual funding, but allows the qualified institutions to submit applications for specific candidates over the next five years. So far, two applications for fellowships have been received for this first round. The applications will be judged

primarily on each candidate's commitment to the field and on his or her academic qualifications. Decisions on the awards will be announced in June, at which point funding will be granted to the appropriate institutions where the fellows will be working.

In accordance with the program's goals, the selected NHF/Baxter fellows will be provided with up to two years of intensive, focused training in hemostasis and thrombosis clinical care and coagulation disorder-related clinical research at the respective institutions. They will also be offered didactic and experiential training in diagnostic and therapeutic procedures, comprehensive care, and clinical research methodology.

For more information about the NHF/Baxter Clinical Fellowship Program, please call Rita Barsky, PhD, Assistant Director of Research, at 212-328-3730, or log on to [www.hemophilia.org](http://www.hemophilia.org).

### **6th Annual Gene Therapy Workshop a Success**

The National Hemophilia Foundation's 6th workshop on Gene Therapies for Hemophilia was held April 25-26, 2003, at The Salk Institute for Biological Studies in La Jolla, California. Approximately 70 researchers from academia, industry, and government met to discuss the latest developments in hemophilia gene transfer research and strategize regarding future directions. Inder Verma, PhD, of The Salk Institute, and Glenn F. Pierce, PhD, MD, of Avigen, Inc, chaired the workshop. Dr. Pierce is a past president of NHF and currently a member of its board of directors.

Participants in the workshop heard presentations on hemophilia and its complications, preclinical models, vectorology, immunology, stem cell therapy, novel approaches, and the status of clinical trials. The event ended with a provocative roundtable discussion of safety and economic issues involving members of the bleeding disorders community, current and former federal regulators, and an expert in informed consent. In addition, NHF grant recipients working in gene therapy presented posters of their work, which were very well received.

The consensus among attendees was that, while progress in gene transfer has slowed due to setbacks in human trials, there is a new clarity and realism among researchers about what the obstacles are to achieving success and what must be done to overcome them. A special section of the September/October issue of *HemAware* will feature more highlights of the conference and the research presented. Additional information, including conference abstracts, will also be available on NHF's Web site, [www.hemophilia.org](http://www.hemophilia.org).

The next workshop will be convened April 23-24, 2004, at The Children's Hospital of Philadelphia.

### **NHF Holds Successful "On the Road" Events in Chicago and Los Angeles**

The first two stops of NHF's 4th Annual NHF on the Road Conference Series took place in Chicago, April 4-5, and Los Angeles, May 2-3. The final stop for the 2003 conference series is in Philadelphia, June 13-14. Each two-day event begins with a Friday training session in prevention education for HTC and chapter staff, and select consumers, followed by a Saturday conference for the local bleeding disorders community. This year's prevention training, as part of the NHF National Prevention Program, targets parents of children with bleeding disorders. The Saturday conference for the local bleeding disorders community includes topics such as the future of treatments, building a resilient family, an update on NHF, breakout sessions covering issues for women with bleeding disorders, young adults, parents and HIV/Hepatitis C, and opportunities to network through rap sessions. Look for photos and a final "On the Road" wrap-up in the September/October edition of *HemAware*.



### **New Board Members. Committee Chairs at NHF**

Four people joined the National Hemophilia Foundation's Board of Directors at a March meeting held in Washington, D.C. The new members are Jerry Hooper, Andra H. James, MD, MPH, Richard Metz, M.D., and Calvin Price. At the same meeting, the board selected its new officers. They are Nikki J. Beneke, PT, Secretary, and Calvin Price, Treasurer. Gina Shreve will continue as President, and will be succeeded next year by Jordan Lurie, MD, who was named President-Elect.

New chairs of NHF committees were also named. The following is a listing of the new chairs by their committee, along with an email address where they may be contacted regarding matters within their respective areas:

Advocacy - Jordan Lurie, MD - [jlurie@hemophilia.org](mailto:jlurie@hemophilia.org)  
 Board Development - Gregory Kerfoot - [gkerfoot@hemophilia.org](mailto:gkerfoot@hemophilia.org)  
 Communications - Steven Faust - [sfaust@hemophilia.org](mailto:sfaust@hemophilia.org)  
 Education - Carletha Gates - [cgates@hemophilia.org](mailto:cgates@hemophilia.org)  
 Finance - Calvin Price - [cprice@hemophilia.org](mailto:cprice@hemophilia.org)  
 MASAC - W. Keith Hoots - [khoots@hemophilia.org](mailto:khoots@hemophilia.org)  
 Resource Development - Stephen Bender - [sbender@hemophilia.org](mailto:sbender@hemophilia.org)

### **Bruce Evatt, MD will be Honored on June 7th at NHF Third Annual Gala in New York**

NHF's third annual gala will be held on Saturday June 7th, at the Starlight Roof in the Waldorf-Astoria Hotel in New York City. Bruce Lee Evatt, M.D., Chief of the Hematologic Disease Branch of the Centers for Disease Control and Prevention, will be honored for his work on behalf of bleeding disorders. Event sponsors include Presenting Sponsor Novo Nordisk, Platinum Sponsors Bayer and Baxter, Gold Sponsor Aventis Behring, and Silver Sponsor Alpha Therapeutic Services. The black tie optional event will begin with cocktails at 7:00pm, to be followed by a short program, dinner and dancing to the Starlight Orchestra. For tickets or more information call Rebecca Schoon at 212-328-3722.

### **NHF's 6th Annual Golf Tour Tees Off**

The 6th Annual NHF Golf Tour, sponsored by Aventis Behring, Banc of America Securities, Baxter and Novo Nordisk,

kicks off at Minisceongo Golf Club, in Pomona, New York, on Monday, June 9th. This great day of golf begins with registration and lunch at 10:30am, a shotgun start at 12noon, followed by cocktails, dinner and the award program at 5:30pm. Additional tournaments are scheduled for selected venues through the fall. At some of the programs, Aventis Behring will sponsor baseball and golf events for youngsters with bleeding disorders. For more information, or a downloadable invitation, please click [here](#) or call Drew Evans at (212) 328-3741 or (800) 42-HANDI x3741.

### **Students and Teachers Raise \$2,000 for the Cure**

Matthew DePace, a student at John Glenn High School in East Northport, NY, raised \$2,000 from his teachers and classmates for NHF's It's Time for A Cure campaign. The school chooses a different charity every year to benefit from proceeds of a particular fund raising event. As a result of Matthew's efforts and many more like him, Phase II of the campaign has already reached the \$3.3 million mark in the first year of a five year campaign with a goal of \$10 million. The first phase of the campaign raised \$5 million on four years - one year ahead of schedule. For more information on the campaign, progress in the search for a cure, and ways to get involved, click [here](#), or read the "Under the Microscope" section in every issue of *HemAware*.